

Muscular Dystrophy - and Much More

Muscular Dystrophies

Duchenne

Becker

Limb-Girdle

Facioscapulohumeral

Congenital

Oculopharyngeal

Distal

Emery-Dreifuss

Myotonic dystrophy



Motor Neuron Disease

Amyotrophic Lateral Sclerosis

Spinal Muscular Atrophy

Spinal Bulbar Muscular Atrophy

Inflammatory Myopathies

Polymyositis

Dermatomyositis

Inclusion Body Myositis

Neuromuscular Junction Diseases

Myasthenia Gravis

Lambert-Eaton Syndrome

Congenital Myasthenic Syndromes

Endocrine Myopathies

Hyperthyroid Myopathy

Hypothyroid Myopathy

Diseases of Peripheral Nerve

Charcot-Marie-Tooth Disease

Friedreich Ataxia

Dejerine-Sottas Disease

Metabolic Myopathies

Phosphorylase Deficiency

Acid Maltase Disease

Phosphofructokinase Deficiency

Debrancher Enzyme Deficiency

Mitochondrial Myopathy

Carnitine Deficiency

Carnitine Palmitoyl Transferase

Deficiency

Phosphoglycerate Kinase

Deficiency

Phosphoglycerate Mutase

Deficiency

Lactate Dehydrogenase Deficiency

Myoadenylate Deaminase

Deficiency

Other Myopathies

Myotonia Congenita

Paramyotonia Congenita

Central Core Disease

Nemaline Myopathy

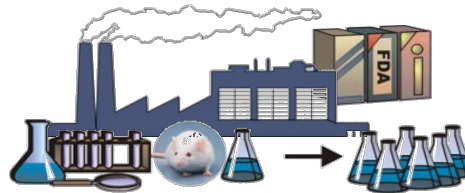
Myotubular Myopathy

Periodic Paralysis

Basic

Translational

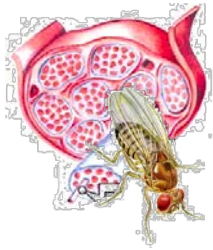
Clinical



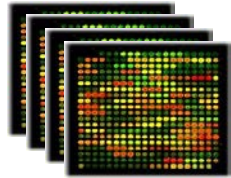
Research Grant Portfolio

- **Basic research grants**
 - **Translational research grants**
 - **Training grants**
 - **Clinical studies / trials grants**
 - **Clinical Research Networks**
(ALS, Duchenne, Myotonic)
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- **Medical Advisory Committee**
 - **Scientific Advisory Committee**
 - **Translational Research Advisory Committee**

MDA Funding Distribution



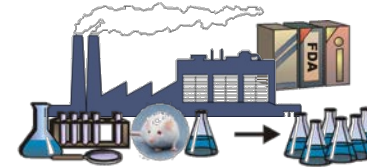
Basic Research



Drug Screening
Target Identification



“Proof-of-Principle”
Testing in Animals



Translational Research
“Preclinical Drug
Development”



Clinical Research
Including Trials

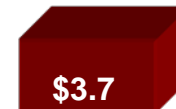
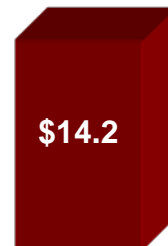
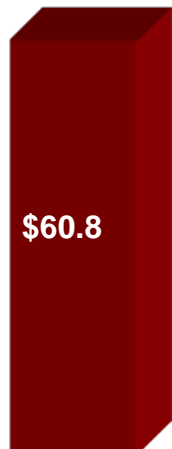
Main Research
Program

Main Research
Program

MVP/ Main Research
Program

MVP

MVP/ Main Research
Program



MDA Funding in Millions

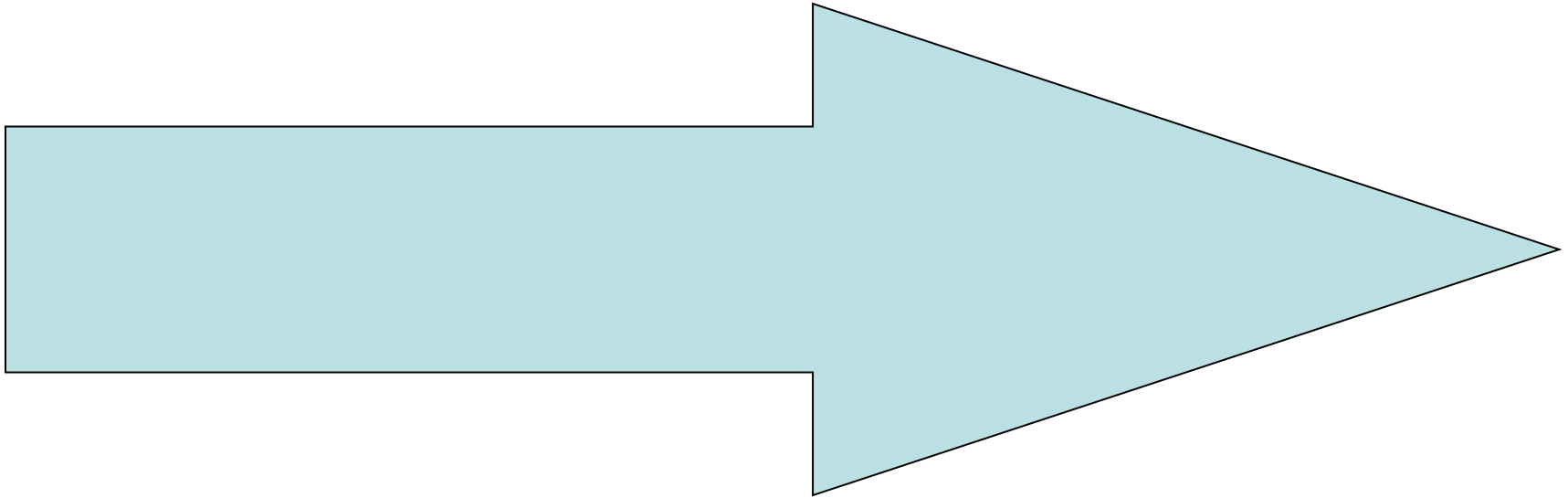
Active projects

Current Gene Therapy Grants

Basic Research	39	\$14,367,139.00
Basic Research - Training	6	\$1,070,631.00
Translational	1	\$369,365.00
Clinical Research Training	1	\$180,000.00
Clinical Research Network	1	\$918,000.00
Clinical Trial	1	\$458,814.00
Meeting	1	\$8,000.00
Total	50 grants	\$17,371,949.00

MDA Invests in Training Researchers....

We are in this for the long haul!



- **Basic researchers**
- **Translational researchers**
- **Clinical researchers**

A call for transparent reporting to optimize the predictive value of preclinical research

Story C. Landis¹, Susan G. Amara², Khusru Asadullah³, Chris P. Austin⁴, Robi Blumenstein⁵, Eileen W. Bradley⁶, Ronald G. Crystal⁷, Robert B. Darnell⁸, Robert J. Ferrante⁹, Howard Fillit¹⁰, Robert Finkelstein¹, Marc Fisher¹¹, Howard E. Gendelman¹², Robert M. Golub¹³, John L. Goudreau¹⁴, Robert A. Gross¹⁵, Amelie K. Gubitz¹, Sharon E. Hesterlee¹⁶, David W. Howells¹⁷, John Huguenard¹⁸, Katrina Kelner¹⁹, Walter Koroshetz¹, Dimitri Krainc²⁰, Stanley E. Lazic²¹, Michael S. Levine²², Malcolm R. Macleod²³, John M. McCall²⁴, Richard T. Moxley III²⁵, Kalyani Narasimhan²⁶, Linda J. Noble²⁷, Steve Perrin²⁸, John D. Porter¹, Oswald Steward²⁹, Ellis Unger³⁰, Ursula Utz¹ & Shai D. Silberberg¹

The US National Institute of Neurological Disorders and Stroke convened major stakeholders in June 2012 to discuss how to improve the methodological reporting of animal studies in grant applications and publications. The main workshop recommendation is that at a minimum studies should report on sample-size estimation, whether and how animals were randomized, whether investigators were blind to the treatment, and the handling of data. We recognize that achieving a meaningful improvement in the quality of reporting will require a concerted effort by investigators, reviewers, funding agencies and journal editors. Requiring better reporting of animal studies will raise awareness of the importance of rigorous study design to accelerate scientific progress.



Links and Contacts

MDA Research Grants:

<http://mda.org/research/guidelines-mda-research-grants-programs>

Insider Tips for Submitting a Successful MDA Research Grant or Development Grant Proposal:

<http://tinyurl.com/cp5vddc>

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